From the INTERNATIONAL SEARCHING AUTHORITY	PCT SEP 2 9 1997
To: CAMPBELL & FLORES Attn. RAMOS, Robert T. 4370 La Jolla Village Drive RECEIVED Suite 700 SAN DIEGO, CALIFORNIA 92122 SEP 2 2 1997 UNITED STATES OF AMERICA CAMPBELL & FLORES CAMPBELL & FLORE	NOTIFICATION OF TRANSMINATION THE INTERNATIONAL SEARCH READER. DATE OR THE DECLARATION (PCT Rule 44.1) Lucy 97
O/Allia	Date of mailing (day month year) 16/09/1997
Applicant's or agent's file reference FP-CE 2563	FOR FURTHER ACTION See paragraphs 1 and 4 below
International application No. PCT/US 97/07725	International filing date (day month year) 08/05/1997
Applicant CEDARS-SINAI MEDICAL CENTER et al.	
1. X The applicant is hereby notified that the International Search Filing of amendments and statement under Article 19: The applicant is entitled, if he so wishes, to amend the claim When? The time limit for filing such amendments is norm International Search Report; however, for more de Where? Directly to the International Bureau of WIPO 34, chemin des Colombettes 1211 Geneva 20, Switzerland Fascimile No.: (41-22) 740.14.35 For more detailed instructions, see the notes on the accompany	s of the International Application (see Rule 46): ally 2 months from the date of transmittal of the stails, see the notes on the accompanying sheet.
2. The applicant is hereby notified that no International Search Article 17(2)(a) to that effect is transmitted herewith. 3. With regard to the protest against payment of (an) additional and the protest against payment.	Il fee(s) under Rule 40.2, the applicant is notified that:
applicants's request to forward the texts of both the pr	n transmitted to the International Bureau together with the cotest and the decision thereon to the designated Offices.
no decision has been made yet on the protest; the appl	icant will be notified as soon as a decision is made.
4. Further action(s): The applicant is reminded of the following: Shortly after 18 months from the priority date, the international a If the applicant wishes to avoid or postpone publication, a notion priority claim, must reach the International Bureau as provided completion of the technical preparations for international publication wishes to postpone the entry into the national phase until 30 m	oe of withdrawal of the international application, or of the line of the cation. In all preliminary examination must be filed if the applicant conths from the priority date (in some Offices even later).
Within 20 months from the priority date, the applicant must perform all designated Offices which have not been elected in the priority date or could not be elected because they are not boun	demand or in a later election within 19 months from the
Name and mailing address of the International Searching Authority European Patent Office, P.B. 5818 Patentlaan 2 NL-2280 HV Rijswijk Tel. (+31-70) 340-2040, Tx. 31 651 epo nl,	Authorized officer A. Overbeeke
Fax: (+31-70) 340-3016	

NOTES TO FORM PCT/ISA/220

These Notes are intended to give the basic instructions concerning the filing of amendments under article 19. The Notes are based on the requirements of the Patent Cooperation Treaty, the Regulations and the Administrative Instructions under that Treaty. In case of discrepancy between these Notes and those requirements, the latter are applicable. For more detailed information, see also the PCT Applicant's Guide, a publication of WIPO.

In these Notes, "Article", "Rule", and "Section" refer to the provisions of the PCT, the PCT Regulations and the PCT Administrative Instructions respectively.

INSTRUCTIONS CONCERNING AMENDMENTS UNDER ARTICLE 19

The applicant has, after having received the international search report, one opportunity to amend the claims of the international application. It should however be emphasized that, since all parts of the international application (claims, description and drawings) may be amended during the international preliminary examination procedure, there is usually no need to file amendments of the claims under Article 19 except where, e.g. the applicant wants the latter to be published for the purposes of provisional protection or has another reason for amending the claims before international publication. Furthermore, it should be emphasized that provisional protection is available in some States only.

What parts of the international application may be amended?

Under Article 19, only the claims may be amended.

During the international phase, the claims may also be amended (or further amended) under Article 34 before the International Preliminary Examining Authority. The description and drawings may only be amended under Article 34 before the International Examining Authority.

Upon entry into the national phase, all parts of the international application may be amended under Article 28 or, where applicable, Article 41.

When?

Within 2 months from the date of transmittal of the international search report or 16 months from the priority date, whichever time limit expires later. It should be noted, however, that the amendments will be considered as having been received on time if they are received by the International Bureau after the expiration of the applicable time limit but before the completion of the technical preparations for international publication (Rule 46.1).

Where not to file the amendments?

The amendments may only be filed with the International Bureau and not with the receiving Office or the International Searching Authority (Rule 46.2).

Where a demand for international preliminary examination has been/is filed, see below.

How?

Either by cancelling one or more entire claims, by adding one or more new claims or by amending the text of one or more of the claims as filed.

A replacement sheet must be submitted for each sheet of the claims which, on account of an amendment or amendments, differs from the sheet originally filed.

All the claims appearing on a replacement sheet must be numbered in Arabic numerals. Where a claim is cancelled, no renumbering of the other claims is required. In all cases where claims are renumbered, they must be renumbered consecutively (Administrative Instructions, Section 205(b)).

The amendments must be made in the language in which the international application is to be published.

What documents must/may accompany the amendments?

Letter (Section 205(b)):

The amendments must be submitted with a letter.

The letter will not be published with the international application and the amended claims. It should not be confused with the "Statement under Article 19(1)" (see below, under "Statement under Article 19(1)").

The letter must be in English or French, at the choice of the applicant. However, if the language of the international application is English, the letter must be in English; if the language of the international application is French, the letter must be in French.

NOTES TO FORM PCT/ISA/220 (continued)

The letter must indicate the differences between the claims as filed and the claims as amended. It must, in particular, indicate, in connection with each claim appearing in the international application (it being understood that identical indications concerning several claims may be grouped), whether

- (i) the claim is unchanged;
- (ii) the claim is cancelled;
- (iii) the claim is new;
- (iv) the claim replaces one or more claims as filed;
- (v) the claim is the result of the division of a claim as filed.

The following examples illustrate the manner in which amendments must be explained in the accompanying letter:

- [Where originally there were 48 claims and after amendment of some claims there are 51]:
 "Claims 1 to 29, 31, 32, 34, 35, 37 to 48 replaced by amended claims bearing the same numbers;
 claims 30, 33 and 36 unchanged; new claims 49 to 51 added."
- [Where originally there were 15 claims and after amendment of all claims there are 11]: "Claims 1 to 15 replaced by amended claims 1 to 11."
- [Where originally there were 14 claims and the amendments consist in cancelling some claims and in adding new claims]:
 "Claims 1 to 6 and 14 unchanged; claims 7 to 13 cancelled; new claims 15, 16 and 17 added." or "Claims 7 to 13 cancelled; new claims 15, 16 and 17 added; all other claims unchanged."
- 4. [Where various kinds of amendments are made]: "Claims 1-10 unchanged; claims 11 to 13, 18 and 19 cancelled; claims 14, 15 and 16 replaced by amended claim 14; claim 17 subdivided into amended claims 15, 16 and 17; new claims 20 and 21 added."

"Statement under article 19(1)" (Rule 46.4)

The amendments may be accompanied by a statement explaining the amendments and indicating any impact that such amendments might have on the description and the drawings (which cannot be amended under Article 19(1)).

The statement will be published with the international application and the amended claims.

It must be in the language in which the international appplication is to be published.

It must be brief, not exceeding 500 words if in English or if translated into English.

It should not be confused with and does not replace the letter indicating the differences between the claims as filed and as amended. It must be filed on a separate sheet and must be identified as such by a heading, preferably by using the words "Statement under Article 19(1)."

It may not contain any disparaging comments on the international search report or the relevance of citations contained in that report. Reference to citations, relevant to a given claim, contained in the international search report may be made only in connection with an amendment of that claim.

Consequence if a demand for international preliminary examination has already been filed

If, at the time of filing any amendments under Article 19, a demand for international preliminary examination has already been submitted, the applicant must preferably, at the same time of filing the amendments with the International Bureau, also file a copy of such amendments with the International Preliminary Examining Authority (see Rule 62.2(a), first sentence).

Consequence with regard to translation of the international application for entry into the national phase

The applicant's attention is drawn to the fact that, where upon entry into the national phase, a translation of the claims as amended under Article 19 may have to be furnished to the designated/elected Offices, instead of, or in addition to, the translation of the claims as filed.

For further details on the requirements of each designated/elected Office, see Volume II of the PCT Applicant's

A. CLASSIFICATION OF SUBJECT MATTER
IPC 6 C12N15/00 C12N15/12 C12Q1/68

GO1N33/577

C12N5/10 A01K67/027 C07K14/47

C07K16/18

According to International Patent Classification (IPC) or to both national classification and IPC

B. FIELDS SEARCHED

 $\begin{array}{ll} \text{Minimum documentation searched} & \text{(classification system followed by classification symbols)} \\ IPC~6~~C12N~~C07K~~C12Q~~G01N~~A01K \end{array}$

Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched

Electronic data base consulted during the international search (name of data base and, where practical, search terms used)

c. Docon	IENTS CONSIDERED TO BE RELEVANT	Relevant to claim No.		
ategory *	Citation of document, with indication, where appropriate, of the relevant passages			
	WO 97 17445 A (CENTRE NAT RECH SCIENT; INST NAT SANTE RECH MED (FR); TORA LAZSLO) 15 May 1997 see page 18, line 25 - page 21, line 14	1-30, 37-43		
(NATURE, vol. 378, no. 6555, 23 November 1995, pages 403-406, XP002009617 TROTTIER Y ET AL: "POLYGLUTAMINE EXPANSION AS A PATHOLOGICAL EPITOPE IN HUNTINGTON'S DISEASE AND FOUR DOMINANT CEREBELLAR ATAXIAS" see the whole document	24,25,39		

 Special categories of cited documents: "A" document defining the general state of the art which is not considered to be of particular relevance 	"T" later document published after the international filing date or priority date and not in conflict with the application but cited to understand the principle or theory underlying the invention
 'E' earlier document but published on or after the international filing date 'L' document which may throw doubts on priority claim(s) or which is cited to establish the publication date of another citation or other special reason (as specified) 'O' document referring to an oral disclosure, use, exhibition or other means 'P' document published prior to the international filing date but later than the priority date claimed 	 "X" document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination being obvious to a person skilled in the art. "&" document member of the same patent family
Date of the actual completion of the international search	Date of mailing of the international search report
28 August 1997	1 6. 09. 97
Name and mailing address of the ISA	Authorized officer
European Patent Office, P.B. 5818 Patentlaan 2 NL - 2280 HV Rijswijk Tel. (+31-70) 340-2040, Tx. 31 651 epo nl, Fax (+31-70) 340-3016	Hornig, H

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Further documents are listed in the continuation of box C.

Patent family members are listed in annex.

REQUEST

The undersigned requests that the present international application be processed

For receiving Office use only
International Application No.
International Filing Date
Name of receiving Office and "PCT International Application"

according to the Patent Cooperation Treaty. Applicant's or agent's file reference FP-CE 2563 (if desired) (12 characters maximum) Box No. I TITLE OF INVENTION NUCLEIC ACID ENCODING SPINOCEREBELLAR ATAXIA-2 AND PRODUCTS RELATED THERETO Box No. II APPLICANT Name and address: (Family name followed by given name; for a legal entity, full official designation. The address must include postal code and name of country.) This person is also inventor. CEDARS-SINAI MEDICAL CENTER 8700 Beverly Boulevard Telephone No. 310.855.5284 Los Angeles, California 90048-1865 United States of America Facsimile No. 310.967.0101 Teleprinter No. State (i.e. country) of nationality: State (i.e. country) of residence: US US the States indicated in the Supplemental Box the United States of America only all designated States all designated States except the United States of America This person is applicant for the purposes of: Box No. III FURTHER APPLICANT(S) AND/OR (FURTHER) INVENTOR(S) Name and address:(Family name followed by given name; for a legal entity, full official designation. The address must include postal code and name of country.) This person is: PULST, Stefan-M. 8125 Skyline Drive applicant only Los Angeles, California 90046 applicant and inventor United States of America inventor only (If this check-box is marked, do not fill in below.) State (i.e. country) of nationality: State (i.e. country) of residence: DE DE all designated States except the United States of America the States indicated in the Supplemental Box all designated States This person is applicant the United States for the purposes of: of America only Further applicants and/or (further) inventors are indicated on a continuation sheet. Box No. IV AGENT OR COMMON REPRESENTATIVE; OR ADDRESS FOR CORRESPONDENCE The person identified below is hereby/has been appointed to act on behalf agent common representative of the applicant(s) before the competent International Authorities as: Name and address: (Family name followed by given name; for a legal entity, full official Telephone No. designation. The address must include postal code and name of country.) 619.535.9001 RAMOS, Robert T. Facsimile No. CAMPBELL & FLORES LLP 619.535.8949 4370 La Jolla Village Drive, Suite 700 San Diego, California 92122 Teleprinter No. United States of America Mark this check-box where no agent or common representative is/has been appointed and the space above is used instead to indicate a special address to which correspondence should be sent.

Box l	No. V	DESIGNATION OF STATES						
The following designations are hereby made under Rule 4.9(a) (mark the applicable check-boxes; at least one must be marked):								
Regional Patent								
	AP ARIPO Patent: KE Kenya, LS Lesotho, MW Malawi, SD Sudan, SZ Swaziland, UG Uganda, and any other State which is a Contracting State of the Harare Protocol and of the PCT							
	EA	A Eurasian Patent: AM Armenia, AZ Azerbaijan, BY Belarus, KG Kyrgyzstan, KZ Kazakstan, MD Republic of Moldova, RU Russian Federation, TJ Tajikistan, TM Turkmenistan, and any other State which is a Contracting State of the Eurasian Patent Convention and of the PCT						
X								
OA OAPI Patent: BF Burkina Faso, BJ Benin, CF Central African Republic, CG Congo, CI Côte d'Ivoire, CM Cameroon, GA Gabon, GN Guinea, ML Mali, MR Mauritania, NE Niger, SN Senegal, TD Chad, TG Togo, and any other State which is a member State of OAPI and a Contracting State of the PCT (if other kind of protection or treatment desired, specify on dotted line).								
Natio	nal P	atent (if other kind of protection or treatment desired,						
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一	AT	Austria	\exists		Madagascar			
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ın add	ution the P	to the designations made above, the applicant also n	ıakes	unde	r Rule 4.9(b) all designations which would be permitted			
		CT except the designation(s) of						

The applicant declares that those additional designations are subject to confirmation and that any designation which is not confirmed before the expiration of 15 months from the priority date is to be regarded as withdrawn by the applicant at the expiration of that time limit. (Confirmation of a designation consists of the filing of a notice specifying that designation and the payment of the designation and confirmation fees. Confirmation must reach the receiving Office within the 15-month time limit.)

Supplemental Box

If the Supplemental Box is not used, this sheet need not be included in the request.

Use this box in the following cases:

1. If, in any of the Boxes, the space is insufficient to furnish all the information:

in particular:

if more than two persons are involved as applicants and/or inventors and no "continuation sheet" is available:

Supplemental Box" is checked:

if, in Box No. II or in any of the sub-boxes of Box No. III, the inventor or the inventor/applicant is not inventor for the purposes of all designated States or for the purposes of the United States of America:

(v) if, in Box No. V, the name of any State (or OAPI) is accompanied by the indication "patent of addition," or "certificate of addition," or if, in Box No. V, the name of the United States of America is accompanied by an indication "Continuation" or "Continuation-in-part":

whose priority is claimed:

2. If the applicant claims, in respect of any designated Office, the benefits of provisions of the national law Disclosures or concerning non-prejudicial disclosures or exceptions to statement below. lack of novelty:

in such case, write "Continuation of Box No. ..." [indicate the number of the Box | and furnish the information in the same manner as required according to the captions of the Box in which the space was insufficient;

in such case, write "Continuation of Box No. III" and indicate for each additional person the same type of information as required in Box No. III:

if, in Box No. II or in any of the sub-boxes of Box in such case, write "Continuation of Box No. II" or "Continuation of No. III, the indication "the States indicated in the Box No. III" or "Continuation of Boxes No. II and No. III" (as the case may be), indicate the name of the applicant(s) involved and, next to (each) such name, the State(s) (and/or, where applicable, ARIPO, Eurasian, European or OAPI patent) for the purposes of which the named person is applicant;

> in such case write "Continuation of Box No. II" or "Continuation of Box No. III" or "Continuation of Boxes No. II and No. III" (as the case may be), indicate the name of the inventor(s) and, next to (each) such name, the State(s) (and/or, where applicable, ARIPO, Eurasian, European or OAPI patent) for the purposes of which the named person is inventor;

if, in addition to the agent(s) indicated in Box No. in such case, write "Continuation of Box No. IV" and indicate for each further agents: in such case, write "Continuation of Box No. IV" and indicate for each further agent the same type of information as required in Box No. IV;

in such case, write "Continuation of Box No. V" and the name of each State involved (or OAPI), and after the name of each such State (or OAPI), the number of the parent title or parent application and the date of grant of the parent title or filing of the parent application;

(vi) if there are more than three earlier applications in such case, write "Continuation of Box No. VI" and indicate for each additional earlier application the same type of information as required in Box No. VI.

in such case, write "Statement Concerning Non-Prejudicial Disclosures or Exceptions to Lack of Novelty" and furnish that

CONTINUATION OF BOX NO. IV:

CAMPBELL, Cathryn IMBRA, Richard J. PERKINS, Susan M. STEINHARDT, Paul C. HOOK, Gregory R. SPOLTER, David I. GAY, David A. FAN, Calvin A. PRESTON, David R. LAUNER, Charlene A.

The above-named attorneys and patent agents are all members of the law firm of Campbell & Flores LLP, and their address is the same as that shown in Box No. IV.

CONTINUATION OF BOX NO. V:

US Continuation-in-Part of 08/727,089 filed 8 October 1996

	PRIORITY (Further priority clair	ns are inc	licated in the Supple	emental Box	
The priority o	of the following e	arlier application	(s) is hereby	claimed:			Sea of Sline	
(in which, or	untry for which, the on was filed)	1	ng Date nonth/year)	Applic	ation No.	(only	ffice of filing for regional or tional application)	
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tem (2)		19 J	19 July 1996					
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tem (3)		8 Oct	ober 1996					
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Box No. IX	SIGNATUR	E OF APPLIC	ANT OR AG	ENT				
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4 Date of	timely receipt of	the required					not receive	
correcti	ons under PCT A	article 11(2):						

Date of receipt of the record copy by the International Bureau:

This sheet is not part of and does not count as a sheet of the international application.

PCT For receiving Office use only FEE CALCULATION SHEET International application No. Annex to the Request Applicant's or agent's **FP-CE 2563** Date stamp of the receiving Office file reference **Applicant** CEDARS-SINAI MEDICAL CENTER CALCULATION OF PRESCRIBED FEES 1. TRANSMITTAL FEE 230.00 Т 2. SEARCH FEE 1,585.00 **EPO** International search to be carried out by (If two or more International Searching Authorities are competent in relation to the international application, indicate the name of the Authority which is chosen to carry out the international search.) 3. INTERNATIONAL FEE **Basic Fee** The international application contains 94 sheets. first 30 sheets 590.00 lb1 \$12.00 768.00 remaining sheets additional amount 1.358.00 Add amounts entered at b₁ and b₂ and enter total at B **Designation Fees** 4 The international application contains _ designations. 572.00 143.00 number of designation fees amount of designation fee payable (maximum 11) 1,930.00 Add amounts entered at B and D and enter total at I (Applicants from certain States are entitled to a reduction of 75% of the international fee. Where the applicant is (or all applicants are) so entitled, the total to be entered at I is 25% of the sum of the amounts entered at B and D.) 0.00 P 4. FEE FOR PRIORITY DOCUMENT 5. TOTAL FEES PAYABLE 3,745.00 Add amounts entered at T, S, I and P, and enter total in the TOTAL box . TOTAL The designation fees are not paid at this time. MODE OF PAYMENT authorization to charge bank draft coupons deposit account (see below) other (specify): cheque cash postal money order revenue stamps DEPOSIT ACCOUNT AUTHORIZATION (this mode of payment may not be available at all receiving Offices) The RO/ $_$ US is hereby authorized to charge the total fees indicated above to my deposit account. is hereby authorized to charge any deficiency or credit any overpayment in the total fees indicated above to my deposit account. is hereby authorized to charge the fee for preparation and transmittal of the priority document to the International Bureau of WIPO to my deposit account. 03-0370 08 May 1997 Deposit Account Number Date (day/month/year) Signature T. Ramos Robert